BMD as a surrogate marker of bone health in children/ adolescents, the prevalence of compromised bone health determined by DXA correlated with symptoms in our cohort demonstrated the value of this objective measure. There is need to systematize BMD evaluations to optimize the opportunities of restoring bone mineral loss during the growing years of pediatric HCT recipients.

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FUTURE RESEARCH NEEDS FOR HEMATOPOIETIC STEM-CELL TRANSPLANT IN PEDIATRICS

Belinson, S.E., Mauger Rothenberg, B., Chopra, R., Aronson, N. Blue Cross Blue Shield Association, Chicago, IL

Objectives: To systematically prioritize research gaps in the areas of HSCT for pediatric malignant solid tumors, inherited metabolic disease, and autoimmune disease. In addition to develop a list of research questions to address the prioritized gaps identified in a comparative effectiveness review of HSCT for pediatrics.
Methods: Central to the methodology of this report was the use of key informants to identify and prioritize evidence gaps. We elicited expert opinion from a group of nine clinical experts, to ensure clinical relevance, a group of patient advocates, and a payer. Through an iterative process, the key informants identified and prioritized research gaps. They then generated and prioritized a list of potential research studies to address these gaps. For the assessment of study designs, we evaluated the appropriateness of a randomized trial, a nonrandomized trial, and a cohort design.
Results: Seven research gaps were identified through a combination of the HSCT CER and conversations with key informants. The patient advocates provided a valuable, unique perspective that resulted in the identification of two additional research gaps and associated research questions. With the patient advocates we intended solely to focus on discussing outcomes. However, the advocates broadened the discussion which in the end coalesced around issues central to the concept of a patient-centered medical home, which could provide the long-term clinical and pharmacy support to help a family from diagnosis to the transition from the treatment center back to their community and then through long-term follow-up. The final prioritized list of gaps is cross-cutting in that each gap applies to more than one indication for HSCT. These appear with their associated key questions, in order of priority in Table 1.
Conclusions: In addition to the prioritized gaps and key questions highlighted by this work, the role of patient advocates, in this case mothers of transplant recipients, was essential to the identification of research gaps along the entire continuum of care from diagnosis to long-term follow-up.

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SAFETY ANALYSIS OF UPPER GASTROINTESTINAL TRACT ENDOSCOPY IN CHILDREN AND ADOLESCENTS AFTER ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION: A FIVE-YEAR, SINGLE-INSTITUTION SURVEY

Ehlert, K.[1], Groll, A.H.[1], Mueller, V.[2], Rossig, C.[1], Juergens, H.[1 1] University Children's Hospital Muenster, Muenster, Northrhine Westfalia, Germany; [2] University Children's Hospital Muenster, Muenster, Northrhine Westfalia, Germany

Indroduction: Upper gastrointestinal tract (GIT) endoscopy is a frequent procedure after allogeneic HSCT, mainly in the context of suspected GvHD. Here, we report our analysis of upper GIT endoscopy in a pediatric, allogeneic HSCT cohort over a period of five years with a special focus on safety aspects.
Patients and Results: From 2007 to 2011, 24 of 86 (28%) pediatric allogeneic HSCT patients had upper GIT endoscopy for various reasons between days +2 and +1079 with a median of day +72. The total number of procedures was 57 with 14 patients having multiple procedures (2-7). The 15 males and 9 females were between 2 to 22 years old (mean 10.2, median 9 years) at the time of their first procedure. Their diagnoses were ALL (11), Fanconi anemia (5), MDS (3), AML (3), Dyskeratosis congenita (1) and Shwachman-Diamond syndrome (1). Transplants were from MSD in 5, from MUD and MMUD in 15 and 4 patients, respectively. The mean platelet count before the procedure was 148000/µl (26000-347000), mean PT 100% (55-130), mean PTT 30sec (23-42) and mean fibrinogen 384mg/dl (113-758). The procedures were primarily performed by pediatric gastroenterology or pediatric surgery and only rarely by adult gastroenterology. The safety analysis included the need of procedure-related initiation or prolongation of mechanical ventilation, blood pressure support, need of transfer to the ICU, GIT wall perforation, occurrence of GIT hemorrhage, and any clinically relevant disturbance of GIT motility. Altogether, procedural adverse events were noted in 1 of 57 procedures (1.8%): A 3-year-old girl had to be transferred to the pediatric ICU on the day of her procedure for life-threatening intestinal hemorrhage at the site of a duodenal biopsy. One day later she also developed a complete duodenal obstruction caused by an intramural duodenal hematoma. Platelet count and coagulation parameters were not different from other patients in this analysis. Over a period of 4 weeks, the obstruction resolved. Possible risk factors in this patient were treatment with defibrotide and disseminated adenovirus disease. All other patients in our analysis had an unremarkable post-procedural course.
Conclusions: Despite of the existence of several potential risk factors, upper GIT endoscopy in children and adolescents after allogeneic HSCT usually is a safe procedure. Intestinal hemorrhage and duodenal wall hematoma with mechanical obstruction is an infrequent, but potentially life-threatening adverse event.

Table 1. RESULTS: Prioritized gaps and key questions

Prioritized Gap	Key Questions
1. Mitigation of long term adverse effects by changes in regimen, including reduced intensity approaches, and changes in subsequent medical or psychosocial intervention.	1.1 Can intense psychological support of patient, parents and siblings prevent development of post-transplant psychological disorders (including PTSD, depression, anxiety, other adverse psychological outcomes) in "surviving" and "non-surviving" family members?
2. Role of novel therapies for HSCT in altering short-term adverse effects and the long-term effects of these therapies. Such approaches include:	2.1 For pediatric patients receiving a transplant due to cancer: Are there interventions that may mitigate immediate and late adverse effects without interfering with the immunotherapeutic effects? 2.2 For pediatric patients receiving a transplant for non-cancerous indications: Are there interventions that may mitigate immediate and late adverse effects without interfering with the establishment and maintenance of chimerism?
3. Impact on outcomes of a "family-centered" approach to transplantation. Advocates of children who have undergone HSCT defined such an approach as including: emotional and psychosocial counseling for the family with a special attention on donor and non-donor siblings, information to share with caregivers and peers, and, provision of tools rather than only a large amount of information for navigating the complexities of the medical system and medication management.	3.1 What approached to integrated care, from diagnosis forward, have the greatest impact in family functioning and overall health and well-being for families faced with pediatric transplant?
4. Effectiveness of survivorship planning on long term, comprehensive follow-up and outcomes.	4.1 Does survivorship planning enhance compliance with long-term follow-up? 4.2 What are the comparative outcomes for those that participate in long-term survivorship follow-up versus those who do not?